CLAIMS

What is claimed is:

1. A receptor-specific liposome for delivering a therapeutic gene to ocular cells, said liposome comprising:

a liposome having an exterior surface and an internal compartment;

a therapeutic gene comprising a sufficient amount of genetic information to encode a therapeutic agent, said therapeutic gene being located within the internal compartment of said nanocontainer;

a plurality of targeting agents comprising blood-retinal barrier and ocular cell membrane targeting agents; and

a plurality of conjugation agents wherein each targeting agent is connected to the exterior surface of said nanocontainer via at least one of said conjugation agents.

2. A receptor-specific liposome according to claim **1** wherein said liposome exterior surface defines a sphere having a diameter of less than 200 nanometers.

3. A receptor-specific liposome according to claim **1** wherein said therapeutic gene is encodes a therapeutic agent selected from the group consisting of genes encoding opsin protein of rhodopsin (RHO), cyclic GMP phosophodiesterase α-subunit (PDE6A) or β-subunit (PDE6B), the alpha subunit of the rod cyclic nucleotide gated channel (CNGA1), RPE65, RLBP1, ABCR, peripherin/RDS, ROM1, arrestin (SAG), alpha-transducin (GNAT1), rhodopsin kinase (RHOK), guanylate cyclase activator 1A (GUCA1A), retina specific guanylate cyclase (GUCY2D), the alpha subunit of the cone cyclic nucleotide gated cation channel (CNGA3), and cone opsin genes such as BCP, GCP, and RCP.

4. A receptor-specific liposome according to claim **1** wherein said therapeutic gene is located within a plasmid.

5. A receptor-specific liposome according to claim **1** wherein the molecular weight of said therapeutic gene is above 30,000 Daltons or wherein said therapeutic gene comprises at least 100 nucleotides.

6. A receptor-specific liposome according to claim **1** wherein between 5 and 1000 targeting agents are conjugated to the surface of said liposome.

7. A receptor-specific liposome according to claim **1** wherein between 25 and 40 targeting agents are conjugated to the surface of said liposome.

8. A receptor-specific liposome according to claim **1** wherein said conjugation agent is selected from the group consisting of polyethylene glycol, sphingomyelin and organic polymers.

9. A receptor-specific liposome according to claim **1** wherein said blood-retinal targeting agent and ocular cell membrane targeting agent is the same targeting agent.

10. A receptor-specific liposome according to claim **1** wherein said targeting agent is selected from the group consisting of insulin, transferrin, insulin-like growth factor (IGF), leptin, low density lipoprotein (LDL), or the corresponding peptidomimetic monoclonal antibodies that mimic these endogenous peptides and bind to the insulin, transferrin, IGF, leptin, or LDL receptor on the blood-retinal barrier and ocular cell membrane.

11. A non-invasive method for delivering a therapeutic gene to ocular cells for expression therein, said method comprising administering to a patient an effective amount of a pharmaceutical preparation comprising:

a) a receptor-specific liposome comprising:

a liposome having an exterior surface and an internal compartment;

a therapeutic gene comprising a sufficient amount of genetic information to encode a therapeutic agent, said therapeutic gene being located within the internal compartment of said liposome;

a plurality of targeting agents comprising blood-retinal barrier and ocular cell membrane targeting agents;

a plurality of conjugation agents wherein each targeting agent is connected to the exterior surface of said liposome via at least one of said conjugation agents; and

b) a pharmaceutically acceptable carrier for said receptor-specific liposome.

12. A non-invasive method according to claim **11** wherein said pharmaceutical preparation is administered intravenously.

13. A non-invasive method according to claim **11** wherein said liposome exterior surface defines a sphere having a diameter of less than 200 nanometers.

14. A non-invasive method according to claim **11** wherein said therapeutic gene is encodes a therapeutic agent selected from the group consisting of genes encoding opsin protein of rhodopsin (RHO), cyclic GMP phosophodiesterase α-subunit (PDE6A) or β-subunit (PDE6B), the alpha subunit of the rod cyclic nucleotide gated channel (CNGA1), RPE65, RLBP1, ABCR, peripherin/RDS, ROM1, arrestin (SAG), alpha-transducin (GNAT1), rhodopsin kinase (RHOK), guanylate cyclase activator 1A (GUCA1A), retina specific guanylate cyclase (GUCY2D), the alpha subunit of the cone cyclic nucleotide gated cation channel (CNGA3), and cone opsin genes such as BCP, GCP, and RCP.

15. A non-invasive method according to claim **11** wherein said therapeutic gene is located within a plasmid.

16. A non-invasive method according to claim **11** wherein the molecular weight of said therapeutic gene is above 30,000 Daltons or 100 nucleotides.

17. A non-invasive method according to claim **11** wherein between 5 and 1000 targeting agents are conjugated to the surface of said liposome.

18. A non-invasive method according to claim **17** wherein between 25 and 40 targeting agents are conjugated to the surface of said liposome.

19. A non-invasive method according to claim **11** wherein said conjugation agent is selected from the group consisting of polyethylene glycol, sphingomyelin, or other organic polymeric substances.

20. A non-invasive method according to claim **11** wherein said blood-retinal barrier targeting agent and ocular cell membrane targeting agent is the same targeting agent.

21. A non-invasive method according to claim **11** wherein said targeting agent is selected from the group consisting of insulin, transferrin, insulin-like growth factor (IGF), leptin, low density lipoprotein (LDL) and the corresponding peptidomimetic monoclonal antibodies that mimic these endogenous peptides and bind to the insulin, transferrin, IGF, leptin, or LDL receptor on the blood-retinal barrier.

22. A non-invasive method according to claim **11** wherein said ocular cell is selected from the group consisting of cells located in the ganglion cell layer (GCL), the inner plexiform layer inner (IPL), the inner nuclear layer (INL), the outer plexiform layer (OPL), outer nuclear

layer (ONL), outer segments (OS) of rods and cones, the retinal pigmented epithelium (RPE), the inner segments (IS) of rods and cones, the epithelium of the conjunctiva, the iris, the ciliary body, the corneum, and epithelium of ocular sebaceous glands.

23. A pharmaceutical preparation comprising:

a) a receptor-specific liposome comprising:

a liposome having an exterior surface and an internal compartment;

a therapeutic gene comprising a sufficient amount of genetic information to encode a therapeutic agent, said therapeutic gene being located within the internal compartment of said liposome;

a plurality of targeting agents comprising blood-retinal barrier and ocular cell membrane targeting agents;

a plurality of conjugation agents wherein each targeting agent is connected to the exterior surface of said liposome via at least one of said conjugation agents; and

b) a pharmaceutically acceptable carrier for said receptor-specific liposome.

24. A pharmaceutical preparation according to claim **23** wherein said blood-retinal barrier targeting agent and said ocular cell membrane targeting agent is the same targeting agent.